THERAPEUTIC DELIVERY COMPOSITIONS AND METHODS OF USE THEREOF

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Abstract of the Invention

The present invention relates to compositions and methods for treating infectious diseases and genetic disorders through gene therapy and intracellular delivery of antisense oligonucleotides or other nucleic acid sequences.

The present invention comprises a therapeutic delivery composition effective for treating a disease state comprising an administerable admixture of an effective amount of a therapeutic compound capable of altering nucleic acid sequence function and an effective amount of a block copolymer having the following general formula:

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or the general formula:

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wherein:

the mean aggregate molecular weight of the portion of the octablock copolymer represented by

polyoxypropylene is between about 5000 and about 7000 Daltons;

a is a number such that the portion represented by polyoxyethylene constitutes between about 10% to about 40% of the compound by weight; and

b is a number such that the polyoxypropylene portion of the total molecular weight of the octablock copolymer constitutes between about 60% and about 90% of the compound by weight.

The present invention also includes compositions and methods using biologically active nonionic reverse block copolymers. The reverse copolymers have an inner core of polyoxypropylene (POP) that is flanked on either end by polyoxyethylene (POE). The reverse block copolymers have the following formula:

$HO(C_3H_6O)_b(C_2H_4O)_a(C_3H_6O)_bH$ **POP POE POP**

wherein "b" represents a number such that the molecular weight of the hydrophobe $(C_3H_6O)_b$ is between approximately 2,000 and 10,000, and "a" represents a number such that the percentage of hydrophile $(C_2H_4O)_a$ is between approximately 5% and 30%.

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